Editas Medicine Announces New EDIT-301 Safety and Efficacy Data in 17 Patients, Presented Today at the American Society of Hematology (ASH) Annual Meeting and in a Company-sponsored Webinar

December 11, 2023

All RUBY patients with ≥5 months follow-up have achieved a normal hemoglobin level and a fetal hemoglobin level of >40%

All patients treated in the RUBY trial are free of vaso-occlusive events post-EDIT-301 infusion

EDIT-301 was well-tolerated and demonstrated a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant

EDIT-301 is now known as renizgamglogene autogedtemcel (reni-cel)

Company-sponsored webinar on the RUBY and EdiTHAL data today at 1:00 p.m. ET; ASH poster presentation today at 9:00 p.m. ET/6:00 p.m. PT

CAMBRIDGE, Mass., Dec. 11, 2023 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage genome editing company, today announced new safety and efficacy data in 17 patients treated with EDIT-301, now known as renizgamglogene autogedtemcel (reni-cel), in the RUBY trial for severe sickle cell disease (SCD) (n=11) and in the EdiTHAL trial for transfusion-dependent beta thalassemia (TDT) (n=6). The total dataset of 17 treated patients includes 12 additional patients since the data presentation at the European Hematology Association (EHA) Annual Congress and in a Company-sponsored webinar this past June. Reni-cel is being investigated in the RUBY and EdiTHAL clinical trials as a potential one-time, durable gene editing medicine for people living with severe SCD and TDT.

Editas Medicine will present the RUBY and EdiTHAL trial data today at 1 p.m. ET in a Company-sponsored **webinar**. The data will also be presented in a poster presentation at the American Society of Hematology (ASH) Annual Meeting in San Diego, CA, at 6:00 p.m. PT (9:00 p.m. ET).

In both the RUBY and EdiTHAL trials to date, reni-cel was well-tolerated and continues to demonstrate a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant by all patients in the two trials (n=17). Since treatment with reni-cel, all RUBY patients are free of vaso-occlusive events (VOEs) (n=11). All RUBY patients with ≥5 months follow-up have maintained a normal hemoglobin level and a fetal hemoglobin level of >40%. All EdiTHAL patients had early and robust increase of total hemoglobin, above the transfusion independence threshold of 9 g/dl (n=6).

“These new and promising data with a larger patient cohort support our belief that reni-cel can be a clinically differentiated, one-time, durable medicine that can provide life-changing clinical benefits to patients with sickle cell disease and beta thalassemia, specifically driving early and robust correction of anemia and sustained increases in fetal hemoglobin,” said Baisong Mei, MD, Ph.D., Senior Vice President and Chief Medical Officer, Editas Medicine. “I would like to thank the clinical trial participants, their families, clinicians, and colleagues at collaborating institutions that contribute to the RUBY and EdiTHAL trials. We look forward to dosing additional patients and sharing further RUBY and EdiTHAL clinical updates in mid-2024.”

“These preliminary results from the RUBY and EdiTHAL trials are encouraging. This investigational gene editing therapy has been well-tolerated and shows promising efficacy, and we look forward to continuing to evaluate its effectiveness on this patient population in need of new treatment options,” said Rabi Hanna, M.D., Chairman of the Division of Pediatric Hematology Oncology and Blood and Marrow Transplantation at Cleveland Clinic Children’s.

Safety
Reni-cel was well-tolerated and demonstrated a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant by all patients in the RUBY and EdiTHAL trials (n=17).

After reni-cel infusion, all treated patients with >2 months follow-up demonstrated successful neutrophil engraftment within one month and platelet engraftment within 1.6 months. No serious adverse events (SAEs) related to reni-cel treatment have been reported.

Efficacy
RUBY Trial in Severe Sickle Cell Disease
In the RUBY trial, all treated patients are free of VOEs since reni-cel infusion. Reni-cel treatment drives early, robust increase of total hemoglobin and fetal hemoglobin. The patients with ≥5 months follow-up have maintained a normal hemoglobin level and a fetal hemoglobin level of >40% (n=6; range 5-18 months follow-up). All treated RUBY patients with >1 month of follow-up followed a similar trajectory of total hemoglobin and fetal hemoglobin increases (n=10).

EdiTHAL Trial in Transfusion-dependent Beta Thalassemia
In the EdiTHAL trial, patients with >1 month follow-up (n=5) demonstrated early and robust total hemoglobin and fetal hemoglobin increases, with total hemoglobin rising above the transfusion independence threshold of 9 g/dL.

Webinar Presentation Details:
The live and archived webcast of the Company’s webinar presentation will be accessible through this webcast link, or through the Events & Presentations page of the “Investors” section of the Company’s website.

A replay of the webinar will be available upon conclusion of the webinar in the Investors section of the Editas Medicine website at https://www.editasmedicine.com/.

ASH Presentation Details:

Title: AsCas12a Gene Editing of *HBG1/2* Promoters with EDIT-301 Results in Rapid and Sustained Normalization of Hemoglobin and Increased Fetal Hemoglobin in Patients with Severe Sickle Cell Disease and Transfusion-Dependent Beta-Thalassemia
Presenting Author: Rabi Hanna, M.D., Department of Pediatric Hematology Oncology and Blood and Marrow Transplantation, Cleveland Clinic Children's, Cleveland, OH, United States
Date/Time: Monday, December 11, 2023, 6:00 – 8:00 p.m. PT/9:00 – 11:00 p.m. ET
Location: San Diego Convention Center, Halls G-H
Session: 801. Gene Therapies: Poster III
Publication Number: 4996

The abstract can be accessed on the ASH website.

About renizgamglogene autogedtemcel (reni-cel)
Reni-cel, formerly known as EDIT-301, is an experimental gene editing medicine under investigation for the treatment of severe sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT). Reni-cel consists of patient-derived $CD34^+$ hematopoietic stem and progenitor cells edited at the gamma globin gene (HBG1 and HBG2) promoters, where naturally occurring fetal hemoglobin (HbF) inducing mutations reside, by AsCas12a, a novel, proprietary, highly efficient, and specific gene editing nuclease. Red blood cells derived from reni-cel $CD34^+$ cells demonstrate a sustained increase in fetal hemoglobin production, which has the potential to provide a one-time, durable treatment benefit for people living with severe SCD and TDT.

About the RUBY Trial
The RUBY trial is a single-arm, open-label, multi-center Phase 1/2 study designed to assess the safety and efficacy of reni-cel in patients with severe sickle cell disease. Enrolled patients will receive a single administration of reni-cel. The RUBY trial marks the first time AsCas12a was used to successfully edit human cells in a clinical trial. Additional details are available on www.clinicaltrials.gov (NCT# 04853576).

About the EdiTHAL Trial
The EdiTHAL trial is a single-arm, open label, multi-center Phase 1/2 study designed to assess the safety and efficacy of reni-cel in patients with transfusion-dependent beta thalassemia. Patients will receive a single administration of reni-cel. Additional details are available on www.clinicaltrials.gov (NCT# 05444894).

About Editas Medicine
As a clinical-stage genome editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas12a and CRISPR/Cas9 genome editing systems into a robust pipeline of treatments for people living with serious diseases around the world. Editas Medicine aims to discover, develop, manufacture, and commercialize transformative, durable, precision genomic medicines for a broad class of diseases. Editas Medicine is the exclusive licensee of Broad Institute's Cas12a patent estate and Broad Institute and Harvard University's Cas9 patent estates for human medicines. For the latest information and scientific presentations, please visit www.editasmedicine.com.

Forward-Looking Statements
This press release contains forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "target," "should," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release include the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including further RUBY and EdiTHAL clinical updates in mid-2024, and the potential of, and expectations for, the Company's product candidates. The Company may not actually achieve the plans, intentions, or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials, including the RUBY and EdiTHAL trials, and clinical development of the Company's product candidates, including reni-cel (EDIT-301); availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; expectations for regulatory approvals to conduct trials or to market products and availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements. These and other risks are described in greater detail under the caption "Risk Factors" included in the Company's most recent Annual Report on Form 10-K, which is on file with the Securities and Exchange Commission, as updated by the Company's subsequent filings with the Securities and Exchange Commission, and in other filings that the Company may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

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Source: Editas Medicine, Inc.